Written Testimony of

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"ARPA-H: The Next Frontier of Biomedical Research"

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Thank you Chair Eshoo, Ranking Member Guthrie, and Members of the Subcommittee on Health for the opportunity to provide input on the proposed Advanced Research Projects Agency for Health (ARPA-H). My name is Esther Krofah, and I am Executive Director of two centers within the Milken Institute, FasterCures and the Center for Public Health.

FasterCures is driven by a singular goal: to save lives by speeding scientific advancements to all patients. With an independent voice, FasterCures is working to build a system that is effective, efficient, and driven by a clear vision: working with our partners to build a patient-centric system where science is accelerated, unnecessary barriers are overcome, and lifesaving and life-enhancing treatments get to those who need them as rapidly and as safely as possible.

FasterCures has long supported giving NIH DARPA-like authorities and capabilities for more high-risk, solutions-oriented R&D, so we are gratified that this concept is being seriously considered at this time.

My comments today will be in the following areas:
    I.   The structure of the proposed new agency, including its location, leadership, authorities, funding and;
    II.   Its activities, including priority-setting and coordination with other agencies and sectors.

I.   <u>STRUCTURE</u>

Location. Some have questioned whether ARPA-H should be housed within NIH, as currently proposed. There is, of course, a more recent example of the DARPA model that has been stood up and can be looked to for lessons learned, and that is ARPA-E (Energy), which resides within the Department of Energy but was meant to serve a purpose and employ an operating model like DARPA's. The experience of ARPA-E should be examined in detail (the <u>National Academies</u> has already done some of that work) and critical success factors identified. We have seen with ARPA-E that an entity like this can exist within a larger federal agency and still foster a different culture and operating model, with the right toolkit and key ingredients. As such, we do not believe this is as critical a threshold question as others might.

We would like to emphasize that this new entity should not be considered a substitute for the National Center for Advancing Translational Sciences (NCATS) at NIH. Some projects of a type

currently pursued by NCATS might fit under the umbrella of ARPA-H, such as platforms like the Tissue Chip or 3-D Tissue Bioprinting programs. But NCATS has such a broad remit to support the whole field and discipline of translational and clinical research that it needs to remain a distinct and well-supported entity.

Leadership. Who leads a new ARPA-H entity will be critical, especially as its first leader, and should be selected for their visionary capacity and their ability to inspire and empower a new team directing milestone-driven initiatives.

ARPA-H will need to ensure expertise from the private sector is engaged both internally and externally. It should ideally have a leader with experience outside academia with a proven track record of success and managing through failures. It will need an external advisory body comprised of patient organizations, industry, academia, and other nonprofits, etc., to inform the agency's priorities.

DARPA's program managers are also a critical asset, and ensuring key people are recruited for those positions is central to the culture change necessary for the success of this effort. This is likely to require freedom from some of the usual constraints of the federal hiring process in order to bring in the right people for limited durations, do it quickly, and pay them appropriately.

Authorities. FasterCures advocated many years ago that NIH be given "Other Transaction Authority (OTA)," like DARPA and other agencies have, to allow for more rapid and flexible engagement with industry and academia to advance projects. NIH does have OTA now, and it is currently being utilized occasionally in NIH initiatives such as Stimulating Peripheral Activity to Relieve Conditions, or SPARC. This is the kind of important tool that will be central to ARPA-H's success.

Funding. Perhaps as or more important than the exact budget number is consistency of funding and sustainability over time. This needs to be a multi-year commitment of effort and funding; it is bigger than a three-year budget line item. A lack of consistent and stable funding for BARDA is arguably one reason the country was not as prepared for the onslaught of COVID-19 as we would have liked to be.

An important reminder for policymakers is that the entire budget of the Food and Drug Administration (FDA) is approximately $6.5 billion. FDA is obviously a critical link in the process that gets exciting new science and products into the hands of patients, and we need to make sure they also have the resources and expertise to keep pace and effectively regulate new technologies coming to them for review through efforts like ARPA-H.

When NCATS was created, there was concern expressed that it would siphon funds away from the other NIH institutes and from basic scientific discovery; as we can see, the opposite has occurred, with significant growth in NIH's budget over the years.

II.　ACTIVITIES

Priorities. ARPA-H should develop a data-driven and science-driven transparent process for setting priorities. For example, a recent HHS effort led by the Office of the Assistant Secretary for Health with the Agency for Healthcare Research and Quality (AHRQ) created a model to

prioritize conditions with high unmet need and low innovation activity in public health. We welcome such a strategic, consistent approach.

In creating this new entity, we should heed key lessons of the COVID-19 pandemic:
- Investment should be prioritized in platform technologies (e.g., mRNA, prototype pathogens) and research infrastructure (e.g., trial networks including community-based networks, data sharing/analytics platforms) that can benefit many researchers and developers.
- A number of high-value platforms and assets were created during the pandemic that should be sustained and made available to ARPA-H (e.g., RADx, the National COVID Cohort Collaborative data platform, NIH's Clinical Trial Capacity Inventory).
- Collaboration, across and within sectors, was key to accelerating research and product development during the pandemic.

Coordination and collaboration. A multi-agency and multi-stakeholder approach should be employed for setting priorities and guiding/informing projects. First, ARPA-H must ensure its work is complementary to and not duplicative of other science-funding agencies such as DARPA, NSF, and BARDA. Second, we strongly believe that representatives of patients, including those from underserved communities, must be a part of priority-setting, defining the problems most important to be solved. And finally, if ARPA-H's work is to be truly solutions-oriented, it must be actively and regularly engaged with and informed by other agencies critical to advancing solutions to patients such as FDA and CMS.

The private sector is a critical partner in this work, whether as collaborators in executing specific projects or as the recipients and amplifiers of any innovation coming out of the program. Technology transfer and commercialization will be central considerations in the policies and approach of the new agency.

Another aspect of working with the private sector could be finding ways to leverage private investment in technologies that might come out of ARPA-H. BARDA Ventures could be a potential model in this regard, or In-Q-Tel, which invests in new technologies related to national security.

Thank you again for the opportunity to offer our input. I am happy to discuss these ideas further and answer any questions you may have.